What You Need to Know About Off-Label Usage of Medications

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What are FDA-approved medications?
The United States Food & Drug Administration (FDA) is responsible for regulating both prescription and non-prescription medications. One of their roles in regulation is to ensure that medications are safe and effective. This is done through the FDA approval process.

What are off-label medications?
This means it is used in a way that is not stated in the FDA labeling. Medications may be used off-label for several reasons such as:

• For a disease or condition that it is not approved to treat. For example, using an antidepressant medication to treat nerve pain.
• At a different dose than approved. For example, a lower or higher dose of the medication may be used for some patients.
• In different dosage form than approved. For example, a medication that is approved for use with an oral tablet but prescribed with an oral solution.

Why are medications used off-label?
Off-label use of medications is common. Although common, many patients may not know that a medication is prescribed off-label. So why are medications used off-label?

One reason may be that the FDA approval process is expensive and time-consuming. If the company would like to add an indication to a medication an additional application is required. For this reason, a medication may still be helpful for off-label use even if it is not approved by the FDA. Off-label use of medications can be particularly useful for patients who have tried all other medications for a disease. Another reason a medication may be prescribed off-label is because there might not be an approved drug to treat a disease. One example is cancer medication. Often a cancer medication may be approved to treat one type of cancer, but is used off-label to treat other types of cancers. Although a medication is not approved for a condition, it may have been studied for its benefits in that condition.

Examples of Off-Label Medication Use for Mental Health Conditions

<table>
<thead>
<tr>
<th>Medication</th>
<th>Indications</th>
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| Amitriptyline | • Insomnia  
• Posttraumatic stress disorder (PTSD) |
| Clonidine | • Smoking cessation  
• Excessive saliva caused by clozapine |
| Gabapentin | • Alcohol dependence  
• Social anxiety |
| Prazosin | • Posttraumatic stress disorder (PTSD) nightmares |
| Topiramate | • Alcohol dependence  
• Weight gain caused by antipsychotic medication  
• Binge eating disorder  
• Bulimia |
| Trazodone | • Insomnia |

Although common, many patients may not be aware that a medication is being prescribed off-label.

Approximately 20% of prescriptions are for an off-label use.

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How does a doctor decide to use a medication off-label?

Once a medicine is approved for one use, doctors can decide whether it is right for other uses. Several factors are considered when a physician decides to prescribe a medication. This includes deciding whether the medication is safe and effective for a specific patient.2,3

Is it safe to take off-label medications?

In most cases, taking a medication off-label is safe. Drug companies are required to prove that a medication is safe for people to use. They just don’t need to prove the drug works for treating an off-label condition. And sometimes a medication could help with symptoms of conditions that it is not approved for. There are many off-label uses that most doctors agree are safe and effective.4

Remember!

- Off-label use means that an approved drug is used in a way that is not approved by the FDA.
- This may include:
  - use of a medication for an unapproved indication or disease
  - use of a medication at an unapproved dose
  - use of a medication in an unapproved dosage form
- Consider asking your doctor about off-label use of medications that you are prescribed.

Go online to learn more about off-label usage of medications, and to view the references for this toolkit.

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